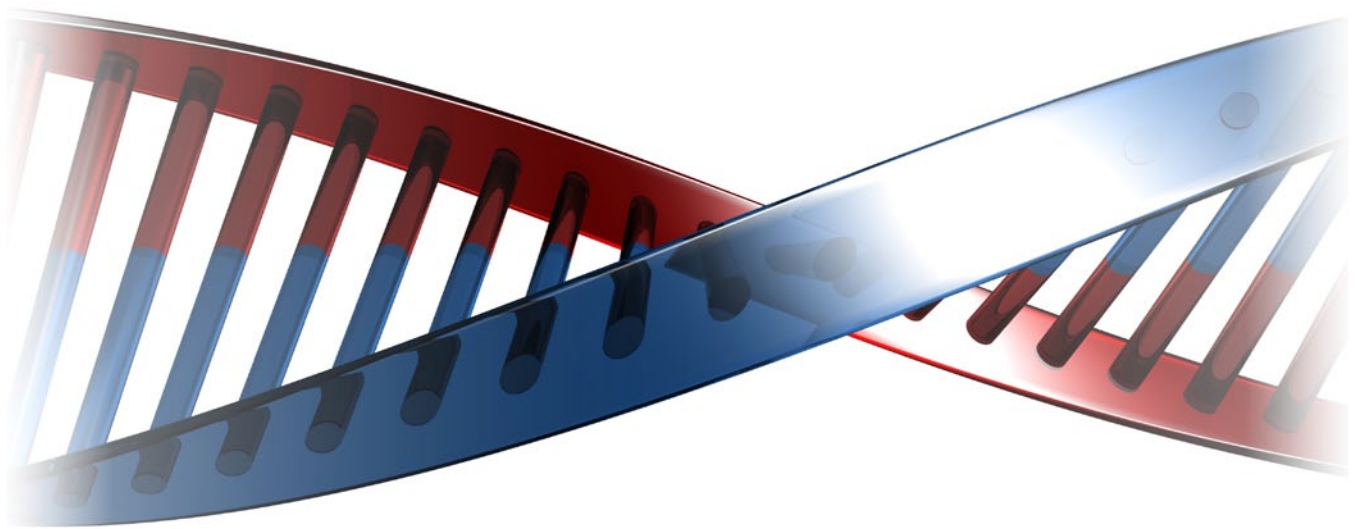


Human Gene Therapies: Novel Product Development

Q&A with Celia M. Witten, Ph.D, M.D.



Celia M. Witten, Ph.D., M.D., is Director of the Office of Cellular, Tissue and Gene Therapy at FDA's Center for Biologics Evaluation and Research (CBER). She worked for more than 10 years as a practicing physician at the National Rehabilitation Hospital in Washington, D.C., before joining FDA as Division Director of General, Restorative, and Neurological Devices in the Center for Devices and Radiological Health. Before attending medical school at the University of Miami, she received a Ph.D. in Mathematics from Stanford University.

Q: What is a human gene therapy product?

A: Human gene therapy is the use of genetic material to treat, cure, or prevent a disease or medical condition.

Q: How do these products work?

A: Genetic material can be used to replace defective genes in a person's body that are responsible for a dis-

ease or medical problem. It can also be used to treat disease through the expression of a protein that the gene codes for. Currently, all gene therapy is investigational.

Q: What is the potential impact of these products for consumers?

A: Research in this area has the potential to revolutionize the treatment of diseases that currently are incur-

able or have inadequate treatments. Gene therapy is being studied to meet unmet medical needs in diverse areas such as genetic diseases, heart disease, cancer, diabetes and stopping the replication of HIV in AIDS patients.

Q: What is FDA's role in regulating these products?

A: Since the first human gene transfer in the late 1980s, CBER has pro-

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vided proactive scientific and regulatory guidance in this area of novel product development.

CBER works closely with product sponsors of potential investigational new drug (IND) applications, and encourages early and frequent dialogue to help define the best scientific approaches and clarify FDA regulatory requirements. These meetings between CBER review staff and product sponsors help reduce product development time and risk.

Q: Have any gene therapy products been approved yet?

A: No. FDA has not yet approved for sale any human gene therapy products.

Q: What are the challenges that scientists and government agencies face in making these products widely available?

A: The development of gene therapy products is a relatively new field compared to other biologics and to traditional drugs. It takes time and careful evaluation for medical interventions based on the administration of genetic material to modify or manipulate the expression of a gene or to alter the biological properties of living cells to develop and potentially result in licensed products.

We continue to see broad interest in the development of a number of different gene therapy vectors used to treat a wide variety of diseases and medical conditions.

Gene therapy is highly innovative and poses some unique and potentially unknown risks. However, it also presents unique possibilities and the

hope to cure and treat diseases in a manner differently, and in some cases potentially better or more safely, than some currently available treatments.

Q: Is FDA conducting or sponsoring these studies?

A: No. As a regulatory agency, FDA's role during the initial review process is to evaluate the information contained in the IND application to verify that safeguards are in place to demonstrate that the rights and welfare of subjects are protected before the study may proceed.

FDA also works closely with our colleagues at the National Institutes of Health and its Recombinant DNA Advisory Committee, academia, and industry to discuss challenges for conducting gene therapy clinical trials.

Q: Studies since the 1980s have involved risks to patients in clinical studies. Can you discuss these risks and what FDA and other entities have done to address them?

A: Although gene therapy products present unknown risks, they also have the potential for tremendous patient benefit. FDA's goal is to minimize those risks.

The agency has continually evaluated its review and oversight processes to improve the conduct of clinical trial research. Efforts to ensure better human subject protection, improve investigator compliance, improve the quality of submitted protocols, and provide additional guidance and standards to facilitate preparation of INDs have been done through educa-

tional outreach, conferences, meetings and policy development.

When developing these new therapies, sponsors of clinical trials must accept responsibility to ensure that participants are not exposed to known unreasonable risks and that the experimental products are as safe as possible. Extensive preclinical studies of safety are typically performed, including in animal models.

Measures are built into trials to protect the safety of each volunteer. However, because these are investigational products and are being used to treat significant illnesses, serious adverse events still may occur during the research process.

CBER is aware of both the promise of gene therapy and the potential for serious adverse events. Patient protection is our paramount concern, and we are committed to minimizing the risks to volunteers who participate in clinical trials of any type, including gene therapy studies.

While the development of promising new experimental therapies presents many challenges, research in this field may lead to the availability of treatments that help save or improve the quality of life for many patients.

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